

# necessity

**NE**w **C**linical  
**E**ndpoints in primary  
**S**jögren's **S**yndrome:  
an **I**nterventional **T**rial  
based on stratif**Y**ing patients

**Primary Sjögren's Syndrome** is the second most prevalent chronic systemic autoimmune disease after rheumatoid arthritis. It typically affects **women** over 40 years old although men may be affected as well. It is also increasingly diagnosed in younger people.

Almost all patients experience extreme **dryness** of the eyes, mouth, skin and genitals, joint **pain** and extreme **fatigue**. About half of patients also experience complications in other organs (e.g. lungs, kidneys). Rarely, patients may develop a type of tumour called lymphoma.

 **9 women / 10 patients**



-  **Dryness (eyes, mouth)**
-  **Lymphoma**
-  **Joint pain**
-  **Fatigue**
-  **Neuropathy**
-  **Vasculitis**
-  **Kidney malfunction**
-  **Lung disease**



The quality of life of patients is **very poor** and unfortunately, there is **no cure** for primary Sjögren's syndrome.

Some treatments improve dryness and pain (such as artificial tears, anti-inflammatory drugs, painkillers), but these are onerous for patients.

There is no treatment for the extreme fatigue which contributes to depression and anxiety. Loss of work productivity and social isolation are very frequent in patients suffering from primary Sjögren's syndrome.

## **Developing new tools to accelerate the discovery of new treatments**

To date, clinical trials in primary Sjögren's syndrome have failed to show that a drug can improve the patient's health. Scientists and clinicians have come to realise that the fact that the **clinical presentation** varies greatly from patient to patient complicates the interpretation of clinical trials. For example, some patients only have dryness, pain and fatigue and no complication in other organs; whereas others have damage in lungs, kidneys and develop lymphoma. These two groups of patients require a different therapeutic approach.

Because a drug may be efficient for some patients, but not for the others, experts realised that clinical trials should analyse data based on **subgroups** including patients with a similar clinical presentation (called stratification of patients).

These subgroups could be based, for example, on disease activity, intensity of a symptom or levels of a particular biomarker in the blood. By analysing these subgroups separately, instead of the whole patient population, we could determine if the drug works for that subset of patients.



The **NECESSITY project** was established to bring to the Sjögren community the necessary **tools** to create subgroups of patients during clinical trials.

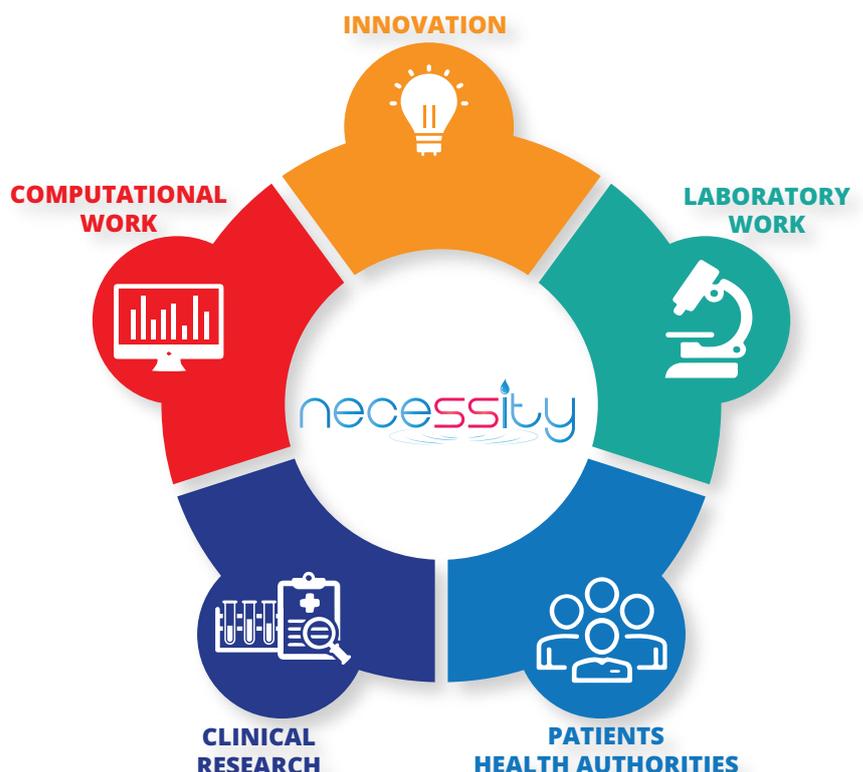
**Develop a new system to score the disease activity and the patient's symptoms**

**Develop new devices and tests to evaluate patients' symptoms and how they feel**

**Discover biomarkers easily detectable in the blood**

The **NECESSITY project** will employ a **multidisciplinary** approach to tackle its ambitious goals. We will use computational work to develop a new scoring system, laboratory work to find new biomarkers and clinical research to develop the new devices and tests to evaluate patient symptoms.

A **clinical trial** will be organised in 8 countries (France, Greece, Italy, the Netherlands, Norway, Spain, Sweden, the United Kingdom) to evaluate the feasibility and usefulness of the new tools developed by the project.



Renowned clinicians, scientists, trialists, statisticians, biologists and patients' representatives have **come together** to form the **NECESSITY Consortium**. It is composed of **25 partners from 10 countries including 20 academic partners, 4 pharmaceutical companies and 1 patient association**.



The project, which officially started on **1<sup>st</sup> January 2019**, is funded by the **“Innovative Medicine Initiative-2 Joint Undertaking”**.

This is a public-private partnership between the European Commission, and the European Federation of Pharmaceutical Industries and Associations (EFPIA).

## **Responding to the unmet needs of patients**

Throughout the project, our strategy will be guided by a constant **interaction** with patients and Health Authorities. The opinions of patients are crucial to identify **underestimated or ignored** aspects of the disease. Patients' views will be provided by the NECESSITY Patient Advisory Group including patients from a number of European countries. Furthermore, discussion with Health Authorities will ensure we obtain their **recommendations** on the development of the new tools.



The impact of the NECESSITY project will be far reaching. New tools and methodologies will be available for **industry** to evaluate new drugs. **Clinical care** will be improved with a better understanding of the disease, a quicker diagnosis, and new therapies to treat and manage the disease. Healthcare delivery needs to support patients will be identified and will lead to the evolution of **public policies**. All these aspects will contribute to improving the quality of life of patients suffering from primary Sjögren's syndrome.

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